majority of the myotubes were stained positive for the dystrophin minigene expression, demonstrating the minigene can be successfully introduced into muscle progenitor cells by retroviral vectors. Such retroviral vector infected progenitor cells or stem cells may be used for the purpose of ex vivo gene therapy for Duchenne and Becker muscular dystrophies.

What is claimed is:

1. An isolated nucleotide sequence encoding a dystrophin minigene comprising:

(a) a N-terminal domain;

(b) four to six rod repeats;

(c) an H1 domain of a dystrophin gene and an H4 domain of the dystrophin gene; and

(d) a cysteine-rich domain,

wherein the N-terminal domain is selected from a group consisting of a N-terminal domain of the dystrophin gene, a modified N-terminal domain of the dystrophin gene, and a N-terminal domain of a utrophin gene; the rod repeats are selected from a group consisting of rod repeats in the dystrophin gene, rod repeats in the utrophin gene, and rod repeats in a spectrin gene; the cysteine-rich domain is a cysteine-rich domain of the dystrophin gene or a cysteine-rich domain of the utrophin gene.

2. The isolated nucleotide sequence of claim 1, further comprising a last three amino acids of a C-terminal domain of the dystrophin gene.

3. An isolated nucleotide sequence encoding a dystrophin minigene comprising:

(a) a N-terminal domain of a dystrophin gene or a modified N-terminal domain of the dystrophin gene;

(b) four to six rod repeats of the dystrophin gene;

(c) an H1 domain of the dystrophin gene and an H4 domain of the dystrophin gene; and

(d) a cysteine-rich domain of the dystrophin gene,

wherein said nucleotide sequence has fewer than 5,000 nucleotides.

4. The isolated nucleotide sequence of claim 3, further comprising an H2 domain of the dystrophin gene and/or an H3 domain of the dystrophin gene.

5. The isolated nucleotide sequence of claim 3, containing four rod repeats of the dystrophin gene.

6. The isolated nucleotide sequence of claim 3, containing five rod repeats of the dystrophin gene.

7. The isolated nucleotide sequence of claim 3, containing six rod repeats of the dystrophin gene.

8. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:2, or a substantially complementary strand of SEQ ID NO:2.

9. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:6, or a substantially complementary strand of SEQ ID NO:6.

10. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:9, or a substantially complementary strand of SEQ ID NO:9.

11. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:10, or a substantially complementary strand of SEQ ID NO:10.

12. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:12, or a substantially complementary strand of SEQ ID NO:12.

13. The isolated nucleotide sequence of claim 3, consisting of SEQ ID NO:14, or a substantially complementary strand of SEQ ID NO:14.

14. A recombinant adeno-associated virus vector, comprising the nucleotide sequence of claim 1 operably linked to an expression control element.

15. The recombinant adeno-associated virus vector of claim 14, wherein the expression control element is an MCK promoter or a CMV promoter.

16. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 8, operably linked to an expression control element.

17. The recombinant adeno-associated virus vector of claim 16, wherein the control element is an MCK promoter or a CMV promoter.

18. A method of treating Duchenne muscular dystrophy and Becker muscular dystrophy in a mammalian subject, comprising:

(i) providing a vector comprising the dystrophin minigene of claim 1 operably linked to an expression control element;

(ii) administering an effective amount of said vector to the mammalian subject under conditions that result in the expression of the dystrophin minigene at a level that provides a therapeutic effect in said mammalian subject.

19. The method of claim 18, wherein the vector is a recombinant adeno-associated virus.

20. The method of claim 18, wherein the vector is a retrovirus.

21. A method of treating Duchenne muscular dystrophy and Becker muscular dystrophy in a mammalian subject, comprising:

(i) providing a vector comprising a dystrophin minigene operably linked to an expression control element;

(ii) administering an effective amount of said vector to the mammalian subject under conditions that result in the expression of the dystrophin minigene at a level that provides a therapeutic effect in said mammalian subject,

wherein the dystrophin minigene has fewer than 5,000 nucleotides comprising:

(a) a N-terminal domain of a dystrophin gene or a modified N-terminal domain of the dystrophin gene;

(b) four to six rod repeats of the dystrophin gene;

(c) an H1 domain of a dystrophin gene and an H4 domain of the dystrophin gene; and

(d) a cysteine-rich domain of the dystrophin gene.

22. The method of claim 21, wherein the vector is a recombinant adeno-associated virus.

23. The method of claim 21, wherein the vector is a retrovirus.

24. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 9, operably linked to an expression control element.

25. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 10, operably linked to an expression control element.

26. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 11, operably linked to an expression control element.

27. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 12 operably linked to an expression control element.

28. A recombinant adeno-associated virus vector, comprising a nucleotide sequence of claim 13, operably linked to an expression control element.